Amicus Therapeutics Announces Approval of Galafold® (Migalastat) for Fabry Disease in Japan

March 23, 2018

First Amicus Medicine and First Oral Precision Medicine for Fabry Patients with an Amenable Mutation in Japan

CRANBURY, N.J., March 22, 2018 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD) announced today that Japan's Ministry of Health, Labour and Welfare (MHLW) has approved the oral small molecule pharmacological chaperone Galafold® capsules 123mg (migalastat) for treatment of patients aged 16 years and older with a confirmed diagnosis of Fabry disease (alpha-galactosidase A deficiency) and who have an amenable mutation. Galafold is the first and only oral precision medicine for Fabry disease in Japan. Amicus will now proceed with pricing and reimbursement processes, and anticipates launching Galafold in Japan in the coming months once those processes have concluded.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “The Japanese approval for Galafold is a major step forward for more than 800 people currently known to be living with Fabry disease in Japan. We believe a significant portion of these Fabry patients have amenable mutations that are suitable for treatment with this differentiated precision oral therapy. I would like to highlight the tremendous collaboration among our Amicus employees, Japanese regulators, and the Fabry community, in particular those physicians and patients who participated in the clinical studies of Galafold and their families who made this approval possible. Japan is very important to our patient-focused vision to provide Galafold to Fabry patients with amenable mutations throughout the world as soon as possible. And now that Amicus has established a strong presence in Japan, we hope that the upcoming Galafold launch will be the first of many future opportunities to deliver new medicines for people living with rare metabolic diseases in Japan.”

Fabry disease is a rare genetic disease and potentially life-threatening condition caused by the accumulation of disease substrate (globotriaosylceramide, GL-3) in the lysosome due to a dysfunctional or deficient enzyme. Galafold works by stabilizing the body's own dysfunctional enzyme, so it can clear the accumulated disease substrate in patients who have amenable mutations. An amenable mutation is one that is responsive to therapy with migalastat based on a proprietary *in vitro* assay (Galafold Amenability Assay).

Prof. Toya Ohashi, Jikei University, stated, “As a principal investigator in the Galafold pivotal studies with extensive experience treating Fabry disease, it can be said that significant unmet need remains. Galafold has a unique mechanism of action that has demonstrated compelling results in naïve and treatment-experienced Fabry patients who have amenable mutations. This differentiated treatment option is good news for the many Fabry patients in Japan who have an amenable mutation.”

Yoshiyuki Suzuki, M.D., Ph.D., Former Professor, International University of Health and Welfare Graduate School, said, “I am pleased with Amicus’ leadership in advancing Galafold to approval. Galafold is part of a chaperone technology platform that originated here in Japan, and represents an important step forward within the field of personalized medicine in Japan.”

Mr. Hisao Harada, Chair of the Japan Fabry Disease Patients and Family Association (JFA) commented, “The approval of Galafold is great news for Fabry patients in Japan as it provides the first new Fabry treatment option in more than a decade. We welcome this oral precision medicine and look forward to it being available to a number of Fabry patients in Japan who have amenable mutations."

An estimated 850 people in Japan are living with Fabry disease. Japan represented approximately 13% of the $1.3B global Fabry ERT sales generated in Japan in 2017.[1] Galafold was approved by the MHLW under the priority review scheme allowed for an Orphan Drug. The approval was based on clinical data from completed clinical studies of Galafold, including two Phase 3 pivotal studies in both treatment naïve (Study 011, or FACETS) and enzyme replacement therapy (ERT) switch patients (Study 012, or ATTRACT), as well as a Phase 1 study that evaluated the pharmacokinetics of migalastat in Japanese volunteers.

Galafold is currently reimbursed in 18 countries, on a commercial basis or through expanded access programs (EAPs). The European Commission granted full approval for Galafold in May 2016 as a first-line therapy for long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease and who have an amenable mutation. Outside the EU, marketing applications have been approved in six markets, including Australia, Canada, Israel, South Korea, Switzerland, and now Japan. Approvals of Galafold are currently pending in the U.S. and Taiwan.

About Galafold and Amenable Mutations

Galafold® capsules 123 mg (migalastat) is a first-in-class chaperone therapy approved in Japan as a monotherapy for Fabry disease in patients with amenable mutations. Galafold works by stabilizing the body’s own dysfunctional enzyme, so it can clear the accumulation of disease substrate in patients who have amenable mutations. A proprietary *in vitro* assay (Galafold Amenability Assay) was used to classify more than 1,000 known *GLA* mutations as “amenable” or “not amenable” to treatment with Galafold. Amicus estimates that 35%-50% of Fabry patients globally may have amenable genetic mutations, and amenability rates within this range vary by geography.

Healthcare providers in the Japan may access the website www.galafoldamenabilitytable.com to quickly and accurately identify which mutations are categorized as “amenable” or “not amenable” to Galafold. Amicus expects to submit additional updates to the website as additional *GLA* mutations are identified and tested in the Galafold Amenability Assay.

Important Japanese Safety Information

Treatment with GALAFOLD should be initiated and supervised by specialists experienced in the diagnosis and treatment of Fabry disease. GALAFOLD is not indicated for use in patients with a nonamenable mutation.

- The efficacy and the safety of concomitant use with enzyme replacement therapy has not been established. GALAFOLD is

not recommended for use in patients with Fabry disease who have severe renal impairment (<30 mL/min/1.73 m^2). The safety and efficacy of GALAFOLD in children 0–15 years of age have not yet been established.
- Migalastat exposure is affected by food, therefore it should not be taken within 2 hours before and after food.
- Patients should be observed carefully, and caution should be taken in the administration in the elderly population.
- If you are pregnant, think you may be pregnant, or are planning to have a baby, do not take this medicine until you have checked with your doctor, pharmacist, or nurse, and consider the treatment only in case that the benefit from migalastat is judged to exceed the risk during pregnancy. Nursing mothers should be instructed not to breast-feed if they are taking migalastat or to discontinue migalastat if they do breast-feed
- Contraindications to GALAFOLD include hypersensitivity to the active substance or to any of the excipients listed in the PRESCRIBING INFORMATION.
- Patients should be monitored based on their course including renal and cardiac function and clinical laboratory test during migalastat treatment. In case no effect is observed in the migalastat treatment, changing treatment should be considered.
- OVERDOSE: General medical care is recommended in the case of GALAFOLD overdose.
- The most common adverse reaction reported was headache, which was experienced by approximately 10% of patients who received GALAFOLD. For a complete list of adverse reactions, please review the Japan package insert.
- Call your doctor for medical advice about side effects.

For further important safety information for Galafold, including dosage and administration, precautions, drug interactions and adverse drug reactions, please see the Japan package insert for Galafold available at http://www.pmda.go.jp/PmdaSearch/iyakuSearch/.

About Fabry Disease
Fabry disease is an inherited lysosomal storage disorder caused by deficiency of an enzyme called alpha-galactosidase A (alpha-Gal A), which is the result of mutations in the *GLA* gene. The primary biological function of alpha-Gal A is to degrade specific lipids in lysosomes, including globotriaosylceramide (referred to here as GL-3 and also known as Gb_3). Lipids that can be degraded by the action of alpha-Gal A are called "substrates" of the enzyme. Reduced or absent levels of alpha-Gal A activity lead to the accumulation of GL-3 in the affected tissues, including the central nervous system, heart, kidneys, and skin. Progressive accumulation of GL-3 is believed to lead to the morbidity and mortality of Fabry disease, including pain, kidney failure, heart disease, and stroke. The symptoms can be severe, differ from patient to patient, and begin at an early age. All Fabry disease is progressive and may lead to organ damage regardless of the time of symptom onset.

About Amicus Therapeutics
Amicus Therapeutics (Nasdaq:FOLD) is a global, patient-centric biotechnology company focused on discovering, developing and delivering novel high-quality medicines for people living with rare metabolic diseases. The cornerstone of the Amicus portfolio is migalastat, an oral precision medicine for people living with Fabry disease who have amenable genetic mutations. Migalastat is currently approved under the trade name Galafold™ in the European Union, with additional approvals granted and pending in several geographies. The lead biologics program in the Amicus pipeline is ATB200/AT2221, a novel, late-stage, potential best-in-class treatment paradigm for Pompe disease. The Company is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases.

[1]Company filings and Amicus estimates

Forward-Looking Statements
This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to approval and commercialization of Galafold in Japan. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with pricing regulatory authorities, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, and the potential that we may not be successful in commercializing Galafold in Japan. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2017. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.

CONTACTS:

Investors/Media:
Amicus Therapeutics
Sara Pellegrino, IRC
Vice President, Investor Relations & Corporate Communications
spellegrino@amicusrx.com
(609) 662-5044

Media:
Pure Communications
Jennifer Paganelli
jpaganelli@purecommunications.com
(347) 658-8290

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